(19) Europäisches Patentamt
European Patent Office
Office européen des brevets

(11) **EP 4 295 913 A3**

(12) **EUROPEAN PATENT APPLICATION**

(88) Date of publication A3:
06.03.2024 Bulletin 2024/10

(43) Date of publication A2:
27.12.2023 Bulletin 2023/52

(21) Application number: **23184106.5**

(22) Date of filing: **20.01.2020**

(51) International Patent Classification (IPC):
A61K 38/16 *(2006.01)* ***A61K 48/00*** *(2006.01)*
A61P 27/16 *(2006.01)* ***C07K 14/00*** *(2006.01)*

(52) Cooperative Patent Classification (CPC):
A61K 38/16; A01K 67/0275; A01K 67/0276; A61K 48/005; A61P 27/16; C07K 14/00;
A01K 2217/056; A01K 2217/075; A01K 2217/20; A01K 2227/105; A01K 2267/0306; C12N 2750/14143; C12N 2750/14144

(84) Designated Contracting States:
AL AT BE BG CH CY CZ DE DK EE ES FI FR GB GR HR HU IE IS IT LI LT LU LV MC MK MT NL NO PL PT RO RS SE SI SK SM TR

(30) Priority: **18.01.2019 EP 19305071**

(62) Document number(s) of the earlier application(s) in accordance with Art. 76 EPC:
20701951.4 / 3 911 354

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(54) AAV-MEDIATED GENE THERAPY RESTORING THE OTOFERLIN GENE

(57) The present inventors report here, in the DFNB9 mouse model (OTOF knock-out mice), the first proof-of-principle that cochlear delivery of a fragmented cDNA via a dual-AAV vector approach can effectively and long-lastingly correct the profound deafness phenotype of these mice when administered well after their auditory system has matured (P30). The present invention therefore concerns a vector system that allows the expression of the full-length Otoferlin polypeptide, or of a functional fragment thereof, in inner hair cells, for use for treating patients suffering from DFNB9 deafness or preventing DFNB9 deafness in patients having DFNB9 mutations, wherein said patients are patients having a developed and mature auditory system, such as new born babies, toddlers, infants, teenagers or adults.

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EUROPEAN SEARCH REPORT

Application Number
EP 23 18 4106

DOCUMENTS CONSIDERED TO BE RELEVANT

Category	Citation of document with indication, where appropriate, of relevant passages	Relevant to claim	CLASSIFICATION OF THE APPLICATION (IPC)
X	HANAN AL-MOYED ET AL: "A dual-AAV approach restores fast exocytosis and partially rescues auditory function in deaf otoferlin knock-out mice", EMBO MOLECULAR MEDICINE (ONLINE), vol. 11, no. 1, 1 January 2019 (2019-01-01), page e9396, XP055574125, DE ISSN: 1757-4684, DOI: 10.15252/emmm.201809396	1,3-15	INV. A61K38/16 A61K48/00 A61P27/16 C07K14/00
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A	AHMED HENA ET AL: "Emerging Gene Therapies for Genetic Hearing Loss", JOURNAL OF THE ASSOCIATION FOR RESEARCH IN OTOLARYNGOLOGY, SPRINGER, NEW YORK, NY, US, vol. 18, no. 5, 16 August 2017 (2017-08-16), pages 649-670, XP036327909, ISSN: 1525-3961, DOI: 10.1007/S10162-017-0634-8 [retrieved on 2017-08-16]	1-15	TECHNICAL FIELDS SEARCHED (IPC) A61K A61P C07K

The present search report has been drawn up for all claims

Place of search	Date of completion of the search	Examiner
Munich	29 January 2024	Vandenbogaerde, Ann

CATEGORY OF CITED DOCUMENTS

X : particularly relevant if taken alone
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A : technological background
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P : intermediate document

T : theory or principle underlying the invention
E : earlier patent document, but published on, or after the filing date
D : document cited in the application
L : document cited for other reasons

& : member of the same patent family, corresponding document

EPO FORM 1503 03.82 (P04C01)